EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

31 March 2016
EMA/819701/2015
Human Medicines Research and Development Support Division

Points to be considered by applicants to the Horizon 2020 topic: New therapies for rare diseases

Work programme 2016-2017: health demographic change and well-being, call: personalised medicine, topic: New therapies for rare diseases (SC1-PM-08–2017)

Brief background information on call SC1-PM-08–2017

- Quote: "Support will be provided to clinical trials on substances where orphan designation has been given by the European Commission, where the proposed clinical trial design takes into account recommendations from protocol assistance given by the European Medicines Agency, and where a clear patient recruitment strategy is presented."
- Dates (announced in 2015): opening 29 July 2016, 1st stage 4 October 2016, 2nd stage 11 April 2017.

Five points to consider before submitting your application for orphan designation and protocol assistance

- Please carefully consider the regulatory timelines for both procedures and plan ahead if you intend incorporating the outlined regulatory requirements into a Horizon 2020 application (see below, Committee for Orphan Medicinal Products (COMP), Scientific Advice Working Party (SAWP)).
- Please establish if your development fulfils the requirements for granting an orphan designation.
- The protocol assistance procedure attracts a fee. Please take this into consideration and investigate any applicable fee reductions, e.g. for SME status.
- It is recommended to request free pre-submission meetings with the EMA Orphan Medicines Office and EMA Scientific Advice Office. This is an opportunity to discuss your draft-application in detail and check the eligibility for starting the respective procedures.
- Please familiarise yourself with the concept of 'significant benefit', one criteria for orphan designation if there are authorised products indicated for your condition. Take this concept into consideration for your development and have a clear plan on how to establish significant benefit for the purpose of orphan designation. Please ask a significant benefit question addressed to COMP

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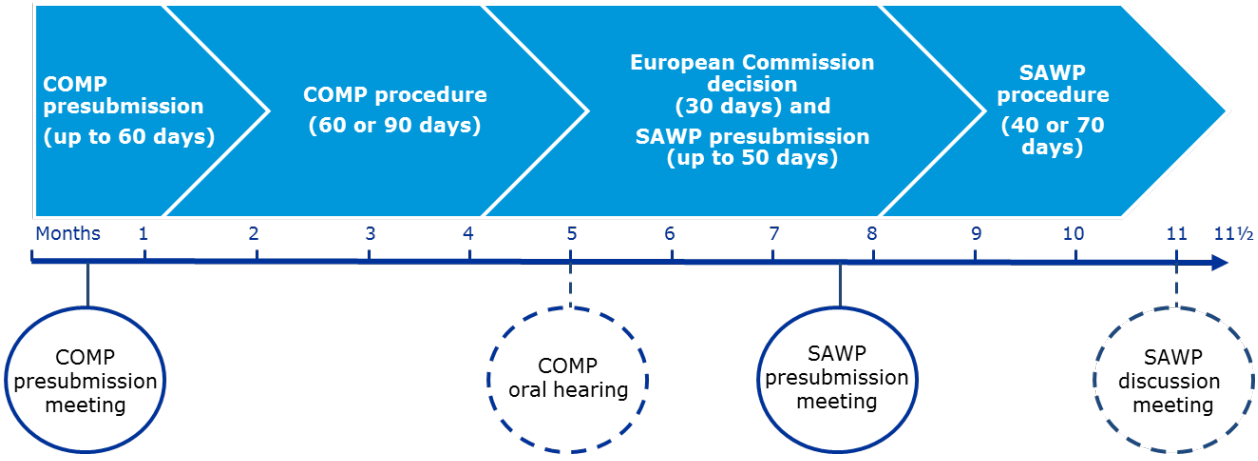
during your protocol assistance procedure for the purpose of establishing significant benefit at the review of orphan designation at the time of marketing authorisation application.

Procedural timeline with potential EMA interaction points

1. COMP orphan designation presubmission phase: 30 days, or 60 days with pre-submission meeting.
2. COMP orphan designation procedure: outcome at procedure day 60, unless oral hearing is required at procedure day 90.
3. European Commission (EC) decision on granting of orphan designation: issued 30 days after COMP opinion.
4. SAWP protocol assistance presubmission phase: 21 days, or 49 days with pre-submission meeting (this step can be started pending the final outcome of the orphan designation).
5. SAWP protocol assistance procedure: final advice letter at procedure day 40, unless discussion meeting is required at procedure day 60 with final advice letter at procedure day 70.



Please contact orphandrugs@ema.europa.eu or scientificadvice@ema.europa.eu with “H2020” in the subject line for any further information.